

Santhera Announces Start of Phase Ib/IIa Trial with POL6014 in Patients with Cystic Fibrosis

Pratteln, Switzerland, October 24, 2018 – Santhera Pharmaceuticals (SIX: SANN) announces the start of a Phase Ib/IIa multiple ascending dose (MAD) trial with POL6014 in patients with cystic fibrosis (CF).

POL6014 is an innovative, potent and selective inhibitor of human neutrophil elastase (hNE) in clinical development for the treatment of CF. The Phase Ib/IIa MAD study is designed to evaluate the safety, tolerability, pharmacokinetics and pharmacodynamics of orally inhaled multiple doses of POL6014 in up to 40 patients with CF. Patients will be treated for 15 days with either placebo or one of three ascending doses of POL6014 given once or twice daily. The trial, which is taking place in sites in Germany and Poland, is expected to complete in 2H 2019.

“In chronic inflammatory conditions of the lung, neutrophils are abundantly present in the tissue and sputum,” said **Marcus Mall**, MD, Professor and Head of Department of Pediatrics, Division of Pneumology, Immunology and Intensive Medicine at the Charité – Universitätsmedizin Berlin. “POL6014 brings new promise to patients with CF, who, despite the introductions of various new treatment options, continue to experience lung tissue inflammation which contributes to pulmonary exacerbations. I am very excited to be part of this Phase Ib/IIa trial, which will further profile POL6014 as an innovative treatment option.”

“POL6014 may be beneficial in treating various pulmonary diseases, where inflammation persists due to elevated neutrophil elastase levels and we are excited to initiate this Phase Ib/IIa trial in patients with CF,” said **Kristina Sjöblom Nygren**, MD, Chief Medical Officer and Head of Development at Santhera. “Santhera will be working with leading experts in CF, as well as the Therapeutics Development Network (TDN) and Clinical Trial Network (CTN), throughout the development of POL6014.”

Santhera obtained the worldwide, exclusive rights from Polyphor AG to develop and commercialize POL6014 in CF and other pulmonary diseases. The Cystic Fibrosis Foundation (CFF) is providing funding support for the conduct of the safety trials with POL6014.

About POL6014

POL6014 is a highly potent and selective inhibitor of human neutrophil elastase (hNE) and was shown to reach high concentrations in the lung when administered by inhalation via an optimized eFlow® nebulizer (PARI Pharma GmbH). A first-in-man Phase I study in healthy volunteers and a single ascending dose (SAD) safety and tolerability Phase Ia study in cystic fibrosis (CF) patients have successfully been completed. Data from the completed Phase Ia study in CF patients has been presented at the North American Cystic Fibrosis Conference (October 18-20, 2018, Denver, CO, USA). The drug candidate was well tolerated and showed evidence of strong elastase inhibition as previously shown in animal models. In addition, POL6014 may show therapeutic benefit for a range of neutrophilic pulmonary diseases with high medical need such as non-cystic fibrosis bronchiectasis (NCFB), alpha-1 antitrypsin deficiency (AATD) or primary ciliary dyskinesia (PCD). POL6014 has EU orphan drug designation (ODD) for the treatment of AATD, PCD and CF.

About Cystic Fibrosis

Cystic fibrosis (CF) is a rare, hereditary, life-threatening, progressive disease affecting approximately 70,000 patients in the U.S. and Europe and is characterized by persistent lung infection and chronic inflammation thereby limiting the ability to breathe over time. Activated or necrotic neutrophils liberate human neutrophil elastase (hNE) in the lung that causes damage to structural, cellular and soluble components of the pulmonary microenvironment. High levels of hNE play a central role in the pathophysiology of CF and correlate with disease severity as measured by functional lung parameters. Inhibition of hNE is expected to stop or slow the damage to lung tissue and may help to improve the overall quality of life for individuals with CF.

About CFF and networks TDN and CTN

The Cystic Fibrosis Foundation (CFF) is the world's leader in the search for a cure, funding promising research and working to provide access to quality, specialized care and treatments for people with CF. www.cff.org. The Therapeutics Development Network (TDN, USA) and Clinical Trial Network (CTN, Europe) are networks of clinical experts which support efficient and patient centric study design, optimized clinical trial execution and high quality data through access to large and experienced CF centers, helping to speed the delivery of new therapies to people with cystic fibrosis.

About Santhera

Santhera Pharmaceuticals (SIX: SANN) is a Swiss specialty pharmaceutical company focused on the development and commercialization of innovative medicines for orphan and other diseases with high unmet medical needs. The portfolio comprises clinical stage and marketed treatments for neuro-ophthalmologic, neuromuscular and pulmonary diseases. Santhera's Raxone® (idebenone) is authorized in the European Union, Norway, Iceland, Liechtenstein and Israel for the treatment of Leber's hereditary optic neuropathy (LHON) and currently commercialized in more than 20 countries. For further information, please visit www.santhera.com.

Raxone® is a trademark of Santhera Pharmaceuticals.

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